CIRM-Funded Clinical Trial for Degenerative Eye Disease Finishes Patient Enrollment

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Oakland, CA – A CIRM-funded clinical trial for retinitis pigmentosa (RP), a degenerative eye disease that causes blindness, recently announced the completion of its patient enrollment for a phase I/IIa study testing a stem cell derived therapy. This is a major step forward in determining whether this approach is both safe and effective at improving sight in RP patients.

RP is a genetically inherited disease that destroys the light-sensing photoreceptor cells at the back of the eye. Symptoms of the disease typically appear in childhood and often cause blindness by the age of 40. RP affects approximately 100,000 people in the US, and there are no effective treatments.

Regenerative medicine offers a promising strategy for treating RP by replacing the lost or damaged photoreceptors in the retina with healthy retinal cells derived from human stem cells.

CIRM is funding a clinical trial that's testing a stem cell-based treatment for advanced RP. The trial is sponsored by a California-based company called jCyte, which was founded in 2012 by Dr. Henry Klassen and Dr. Jing Yang, both currently professors at UC Irvine.

The treatment involves injecting human retinal progenitor cells, which are derived from adult stem cells, into the damaged area of the retina at the back of the eye to hopefully improve vision. These progenitor cells could either replace the damaged photoreceptors in the eye, or could help rescue the remaining photoreceptors from being destroyed.

Earlier this year, jCyte reported that they had treated the first nine patients in their phase I/IIa safety trial and did not observe any negative side effects caused by the treatment. Today, they announced that they have finished the trial enrollment with a total of 28 patients. Four different doses of retinal progenitor cells were tested in this patient group to determine both safety and the optimal dose of cells. While the results of this trial won't be available until next year, eight of the enrolled patients have already completed the one-year study and have shown promising safety results.

"We have successfully completed four DSMB (Data Safety Monitoring Board) reviews," said Dr. Klassen. "So far, trial participants have had no significant side effects, with good tolerance of the injected cells. We are quite gratified by the results."

CIRM is also happy to hear these positive findings as proving that a stem cell treatment is safe in patients is essential for moving a clinical trial forward.

"We are really encouraged by the preliminary safety results of the jCyte trial," says Jonathan Thomas, Ph.D., J.D., Chair of the CIRM Governing Board. "RP is a rare disease and an unmet medical need that could benefit from advances in stem cell-based treatments. The jCyte trial will hopefully pave the way for determining how stem cells can improve vision in RP patients, and ultimately other diseases of blindness."

As this trial moves forward, jCyte hopes to begin planning a phase IIb trial that will determine whether their stem cell-based therapy is effective at improving vision in advanced RP patients.

"I look forward to the next stage of development towards commercialization," said jCyte CEO Paul Bresge in a news release. "We never lose sight of our singular goal: to ultimately deliver this much-needed therapy to patients."

If all goes well, additional RP patients will be needed to participate in the second phase of the jCyte trial. Patients who are interested in learning more about this trial or enrolling in future trials, should visit the jCyte website.

About CIRM

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With $3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information, go to www.cirm.ca.gov.
